

Elucidating pathways from hereditary Alzheimer mutations to pathological tau phenotypes

Grant Award Details

Elucidating pathways from hereditary Alzheimer mutations to pathological tau phenotypes

Grant Type: Basic Biology V

Grant Number: RB5-07011

Project Objective: To identify new mechanisms and pathways that tie mutations causing Alzheimer's Disease (AD) to validated endpoints of AD in bona fide human neurons.

Investigator:

| | |
|---------------------|-------------------------------------|
| Name: | Lawrence Goldstein |
| Institution: | University of California, San Diego |
| Type: | PI |

Disease Focus: Alzheimer's Disease, Neurological Disorders

Human Stem Cell Use: iPS Cell

Cell Line Generation: iPS Cell

Award Value: \$1,050,300

Status: Closed

Progress Reports

Reporting Period: Year 1

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Reporting Period: Year 2

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Reporting Period: Year 3

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Grant Application Details

Application Title: Elucidating pathways from hereditary Alzheimer mutations to pathological tau phenotypes

Public Abstract: We propose to elucidate pathways of genes that lead from early causes to later defects in Alzheimer's Disease (AD), which is common, fatal, and for which no effective disease-modifying drugs are available. Because no effective AD treatment is available or imminent, we propose to discover novel genetic pathways by screening purified human brain cells made from human reprogrammed stem cells (human IPS cells or hiPSC) from patients that have rare and aggressive hereditary forms of AD. We have already discovered that such human brain cells exhibit a unique biochemical behavior that indicates early development of AD in a dish. Thus, we hope to find new drug targets by using the new tools of human stem cells that were previously unavailable. We think that human brain cells in a dish will succeed where animal models and other types of cells have thus far failed.

Statement of Benefit to California: Alzheimer's Disease (AD) is a fatal neurodegenerative disease that afflicts millions of Californians. The emotional and financial impact on families and on the state healthcare budget is enormous. This project seeks to find new drug targets to treat this terrible disease. If we are successful our work in the long-term may help diminish the social and familial cost of AD, and lead to establishment of new businesses in California using our approaches.

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